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'Miracle' gene therapy trial halted

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A "miracle" gene therapy treatment for children suffering from the fatal "bubble boy" disease has been halted in France, after one of the patients developed leukaemia as a direct consequence of the treatment. However, British doctors argue that without the treatment many of the patients are certain to die, and say a similar trial in UK will continue.

Boys with X-SCID (Severe Combined Immunodeficiency) have a faulty copy of a gene on their X chromosome that makes an immune protein called interleukin-2. As a result, they have no resistance to infection and die unless treated.

In 2000, a team led by Alain Fischer at Necker Hospital, Paris, carried out the first gene therapy treatment, which replaced the faulty gene. It was one of only a handful of successful gene therapy trials in people. In April 2002, the mother of a Welsh boy treated at Great Ormond Street hospital in London described his progress as "nothing short of a miracle".

A total of 15 patients, have been treated so far - 11 in Paris and four in London. But it was revealed on Thursday that one three-year-old patient has now developed leukaemia.

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The boy underwent gene therapy at the age of six months, and contracted chicken pox at two-and-a-half. His white cell count increased in response to the infection, as would be expected. But his bone marrow then started uncontrollably producing these cells.

Gene therapy involves shuttling the gene into a patient's cells using a harmless virus. But transferred genes cannot be targeted to insert into a specific part of a chromosome. And it appears, scientists say, that in this boy the new gene was inserted next to an oncogene, called Lmo2, triggering the leukaemia.

This kind of severe side-effect was not unanticipated. "In its original review of these studies, the UK Gene Therapy Advisory Committee recognised th possibility of cancer occurring as a result of

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this typ of gene therapy," says Norman Nevin, GTAC's chairman.

But Nevin confirmed that the UK trials will continue: "GTAC is satisfied that all parents and children treated were informed of this risk and received appropriate counselling prior to treatment." The chances of the gene being inserted next to an oncogene are very low, he adds.

Some children with X-SCID are candidates for bone marrow transplant treatments, but others will undoubtedly die without gene therapy. Doctors at Great Ormond Street say two UK patients have died in 2002, because they did not start gene therapy in time. "It is ethically justifiable to go ahead with the Great Ormond Street trials because of the benefits accrued from the treatment," says Nevin.

The full investigation into the three-year-old's leukaemia will take between 12 and 18 months. Doctors say he is responding well to chemotherapy.

Emma Young

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